

ADENO-ASSOCIATED VIRUS VECTORS
ENCODING FACTOR VIII AND METHODS OF USING THE SAME

ABSTRACT

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The present invention provides recombinant adeno-associated virus (rAAV) vectors comprising a heterologous nucleotide sequence encoding factor VIII (factor VIII). In preferred embodiments, the factor VIII is a B-domain deleted factor VIII. Also provided are methods of producing a high titer stock of the inventive rAAV/factor VIII
10 vectors. Another aspect of the invention is a method of delivering a nucleotide sequence encoding factor VIII to a cell, preferably for subsequent administration to a subject. The present invention further provides methods of administering rAAV/factor VIII to a subject, *e.g.*, for the treatment of hemophilia. The rAAV vector may be administered by any route, but is preferably administered to the liver.